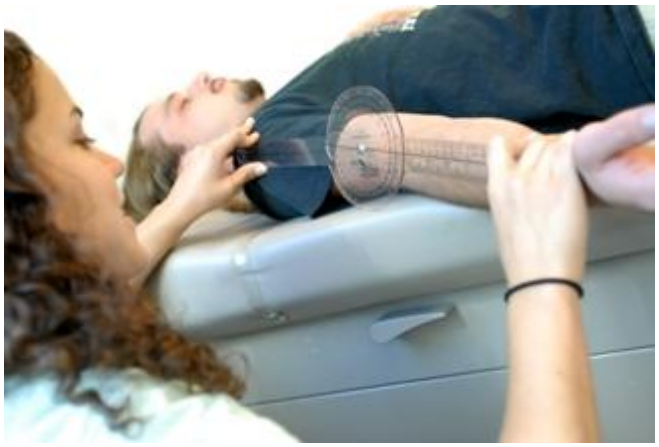
NCBDDDannualreport2011
National Center on Birth Defects and Developmental Disabilities • Centers for Disease Control and Prevention

Hemophilia and Von Willebrand Disease

Preventing and Controlling Complications Resulting From Bleeding Disorders



What is the problem?

- Bleeding disorders such as hemophilia, and von Willebrand Disease (VWD) can lead to spontaneous internal bleeding and bleeding following injuries and surgery. Bleeding into joints can cause crippling and chronic pain if not appropriately treated with replacement blood products to facilitate blood clot formation.
- About 15% to 20% of people with hemophilia develop an inhibitor that results in the body's resistance to treatment products used to treat their bleeding disorder. Treatment for patients with inhibitors is extremely difficult and the cost of care can skyrocket. Patients with inhibitors often experience increased joint disease and other complications from bleeding that result in a reduced quality of life.
- VWD is an under-diagnosed blood disorder in which the blood does not clot properly.
- Current data estimate that as many as 1 percent of women in the United States may have a bleeding disorder, and many are unaware of their condition.
- Women with heavy menstrual bleeding (menorrhagia) and/or VWD are at increased risk for anemia, pain during menstruation, hospitalizations, blood transfusions, limitations in daily activities, time lost from work or school, and a reduced quality of life.

What do we know?

- Hemophilia affects 1 in 5,000 male births. About 400 babies are born with hemophilia each year.
- Currently, about 20,000 males in the United States have hemophilia.
- About half of those affected by hemophilia have the severe form. Hemophilia affects people from all racial and ethnic groups.
- People with hemophilia who develop an inhibitor are twice as likely to be hospitalized for a bleeding complication.

- VWD is the most common bleeding disorder, found in up to 1% of the U.S. population. This means that 1.4 million (or about 1 in every 100) people in the United States have the disease. Although VWD occurs among men and women equally, women are more likely to notice the symptoms because of heavy or abnormal bleeding during their menstrual periods and after childbirth.

- Although there is no cure for VWD, treatment can control symptoms and help people avoid problems.

- Most people who have VWD are born with it. It is almost always inherited, passed down from either the mother or the father, or both, to the child.

What can we do?

- Continue to support education and outreach activities to prevent secondary conditions in people with bleeding disorders by improving their access to public health programs and implementing effective health promotion and wellness programs.

- Enhance laboratory research capacity in the community by providing collaborating investigators with services such as subject matter expertise, technical support, and laboratory analysis.

- Increase provider and patient education and awareness of the clinical benefit of routine inhibitor screening.

- Increase public health research of bleeding disorders outcomes and risks for complications such as joint disease, and inhibitor development to improve care and prevention of morbidity.

- Begin tracking for inhibitor development and associated morbidity.

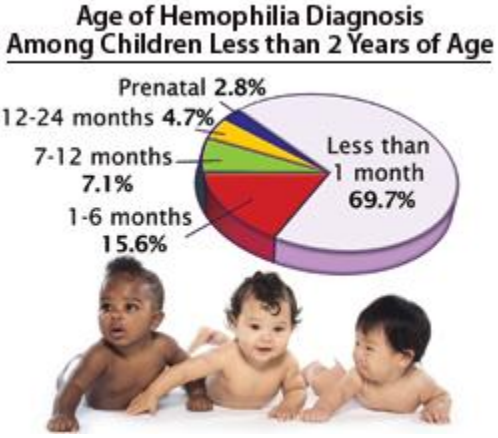
- Continue tracking patients with bleeding disorders to better understand bleeding disorders and their complications.

- Assess women's awareness and knowledge of bleeding disorders and assess OB/GYN knowledge and practice related to bleeding disorders.

Accomplishments

- Created and made available for public use on CDC's website, CHAMP (CDC Hemophilia A Mutation Project) a database of more than 2000 unique hemophilia mutations reported worldwide. Knowing the mutation a person with hemophilia has is important for genetic testing of family members and may help in predicting how likely a person is to develop an inhibitor and, in the future, predict what treatments might work better for different mutations.

- Presented information on women and bleeding disorders such as VWD as part of CDC's Expert Video Commentary Series.



- Developed "Introduction to Hemophilia Care" an online course that promotes a family-centered approach in the diagnosis, assessment and management of hemophilia and addresses potential complications from hemophilia and its treatment. The course is available on our website, is free of charge and provides NCE/CEU credit.

- Developed a dataset of normal joint range of motion (ROM) measurements. The dataset can be used by researchers in comparison studies and to study patterns of joint ROM changes in patient populations with chronic joint disease due to other disorders such as rheumatoid arthritis.

- Working with the Hemophilia Federation of America, highlighting physical activities and dietary meal plans based on age, weight, and medical complications secondary to hemophilia which are components of the FitFactor: Strength, Flexibility, and Wellness program.

- Working with the National Hemophilia Foundation, launched the Steps for Living Website, a one-stop resource for information on bleeding disorders for kids, adolescents, parents and health educators to promote healthy living for the whole family.

- Completed formative research, product development, and message testing to identify materials and messages that will help children and adolescents with hemophilia maintain or improve their health as they become more independent and move toward adulthood. This research resulted in the production of two videos for children with hemophilia focused on disclosure and safe sports.

Did you know?

- For the one-third of babies born with hemophilia in families with no known history of hemophilia, the diagnosis is made when an unusual bleeding event occurs. The most common sites of bleeding are the circumcision site and the head.
- Over 27,000 individuals have participated in our Universal Data Collection (UDC) system, providing information and blood specimens that are used to measure rates of complications of bleeding disorders, identify issues that require further research, and investigate new blood-borne threats.
- Youth with hemophilia are just as likely as youth among the general population to be overweight. However, they are more likely to have decreased joint mobility than those who are not.

Looking to the future

- Increase the focus on preventing and controlling complications resulting from bleeding disorders.

- Conduct pilot tracking projects among patients receiving care outside of the federally funded hemophilia treatment centers to better understand public health needs and gaps of all patients with bleeding disorders.

- Continue analysis of tracking data collected through the Universal Data Collection (UDC) program.

- Continue to build upon the data that has been collected through the UDC and expand and improve bleeding disorders tracking to include tracking of current and emerging issues such as aging, inhibitor development.
- Conduct an epidemiologic research study to better understand the risks and implications of cardiovascular disease among persons with hemophilia.
- Conduct research to better understand development and prevention of joint disease among persons with hemophilia.
- Host a panel of experts to develop recommendations for screening practices for inhibitor development in people with hemophilia.
- Increase provider and patient awareness of the clinical benefit of routine inhibitor screening.
- Publish findings from the Harris Interactive internet survey that assessed women's level of knowledge about bleeding disorders.
- Publish findings from the formative research and message testing that was conducted to identify transition materials and messages needed by the hemophilia community.

Featured Video

View "A Look at Hemophilia" Video

Notable 2011 NCBDDD Scientific Publications

- Baker JR, Riske B, Voutsis M, Cutter S, Presley R. Insurance, Home Therapy, and Prophylaxis in U.S. Youth with Severe Hemophilia. American Journal of Preventive Medicine 2011;41(6S4):S338-45.
- Kulkarni R, Soucie JM. Pediatric hemophilia: a review. Semin Thromb Hemost. 2011; 37(7):737-44.
- Monahan PE, Baker JR, Riske B, Soucie JM. Physical Functioning in Boys with Hemophilia in the U.S. American Journal of Preventive Medicine 2011;41(6S4):S360-68.
- Shapiro AD, Soucie JM, Peyvandi F, Aschman DJ, DiMichele DM, on behalf of the UDC Rare Bleeding and Clotting Disorders Working Group and the European Network Rare Bleeding Disorders Database. Knowledge and Therapeutic Gaps: A Public Health Problem in the Rare

Coagulation Disorders Population. American Journal of Preventive Medicine 2011;41(6S4):S324-31.

- Sharathkumar AA, Soucie JM, Trawinski B, Griest A, Shapiro AD. Prevalence and risk factors cardiovascular disease (CVD) events among patients with haemophilia: experience of a single haemophilia treatment centre in the United States (US). Haemophilia 2011; 17:597-604.
- Soucie JM, Wang C, Forsyth A, Funk S, Denney M, Roach KE, Boone D, and the Hemophilia Treatment Center Network. Range of motion measurements: reference values and a database for comparison studies. Haemophilia 2011; 17:500-7.
- Soucie JM, Wang C, Siddiqi A, Kulkarni R, Recht M, Konkle BA, and the Hemophilia Treatment Center Network. The longitudinal effect of body adiposity on joint mobility in young males with haemophilia A. Haemophilia 2011; 17:196-203.
- Witmer C, Presley R, Kulkarni R, Soucie JM, Manno CS, Raffini L. Associations between intracranial hemorrhage and prescribed prophylaxis in a large cohort of hemophilia patients in the United States. Br J Haematol 2011; 152:211-216.
- Miller CH, Benson J, Ellingsen D, Driggers J, Payne A, Kelly FM, Soucie JM, Hooper WC and the Hemophilia Inhibitor Research Study Investigators. F8 and F9 mutations in US haemophilia patients: correlation with history of inhibitor and race/ethnicity. Haemophilia 2011; 18:375-82.